
New Study Shows CIRM-Supported Therapy Cures More than 95% of Children Born with a Fatal Immune Disorder.

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Oakland, CA – A study published in the New England Journal of Medicine shows that an experimental form of stem cell and gene therapy has cured 48 of 50 children born with a deadly condition called ADA-SCID.

Children with ADA-SCID, (severe combined immunodeficiency due to adenosine deaminase deficiency) lack a key enzyme that is essential for a healthy, functioning immune system. As a result, even a simple infection could prove fatal to these children and, left untreated, most will die within the first two years of life.

In the study, part of which was supported by CIRM, researchers at the University of California Los Angeles (UCLA) and Great Ormond Street Hospital (GOSH) in London took some of the children's own blood-forming stem cells and, in the lab, corrected the genetic mutation that causes ADA-SCID. They then returned those cells to the children. The hope was that over time the corrected stem cells would create a new blood supply and repair the immune system.

In the NEJM study the researchers reported outcomes for the children two and three years post treatment.

"Between all three clinical trials, 50 patients were treated, and the overall results were very encouraging," said Dr. Don Kohn, a distinguished professor of microbiology, immunology and molecular genetics at the David Geffen School of Medicine at UCLA and a member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. "All the patients are alive and well, and in more than 95% of them, the therapy appears to have corrected their underlying immune system problems."

Two of the children did not respond to the therapy and both were returned to the current standard-of-care therapy. One subsequently underwent a bone marrow transplant. None of the children in the study experienced serious side-effects.

"This is encouraging news for all families affected by this rare but deadly condition," says Maria T. Millan, MD, President and CEO of CIRM. "It's also a testament to the power of persistence. Don Kohn has been working on developing this kind of therapy for 35 years. To see it paying off like this is a remarkable testament to his skill as a researcher and determination to help these patients."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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